



4164-01-P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2019-N-5646]

Food and Drug Administration Rare Disease Day 2020: Supporting the Future of Rare Disease Product Development; Public Meeting; Request for Comments

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice of public meeting; request for comments.

SUMMARY: The Food and Drug Administration (FDA or the Agency) is announcing a public meeting and an opportunity for public comment on "FDA Rare Disease Day 2020: Supporting the Future of Rare Disease Product Development." Developing a treatment for a rare disease can present unique challenges. The goal of this meeting is to obtain stakeholders' perspectives on challenges and solutions in rare disease product development and identify commonalities that can support product development across a variety of rare diseases.

DATES: The public meeting will be held on February 24, 2020, from 9 a.m. to 5 p.m. Submit either electronic or written comments on the public meeting by March 29, 2020. See the SUPPLEMENTARY INFORMATION section for registration date and information.

ADDRESSES: The public meeting will be held at the FDA White Oak Campus, 10903 New Hampshire Ave., Building 31 Conference Center, the Great Room (Rm. 1503), Silver Spring, MD 20993-0002. Entrance for the public meeting participants (non-FDA employees) is through Building 1, where routine security check procedures will be performed. For parking and security information, please refer to

<https://www.fda.gov/about-fda/white-oak-campus-information/public-meetings-fda-white-oak-campus>.

You may submit comments as follows. Please note that late, untimely filed comments will not be considered. Electronic comments must be submitted on or before March 29, 2020. The <https://www.regulations.gov> electronic filing system will accept comments until 11:59 p.m. Eastern Time at the end of March 29, 2020. Comments received by mail/hand delivery/courier (for written/paper submissions) will be considered timely if they are postmarked or the delivery service acceptance receipt is on or before that date.

Electronic Submissions

Submit electronic comments in the following way:

- Federal eRulemaking Portal: <https://www.regulations.gov>. Follow the instructions for submitting comments. Comments submitted electronically, including attachments, to <https://www.regulations.gov> will be posted to the docket unchanged. Because your comment will be made public, you are solely responsible for ensuring that your comment does not include any confidential information that you or a third party may not wish to be posted, such as medical information, your or anyone else's Social Security number, or confidential business information, such as a manufacturing process. Please note that if you include your name, contact information, or other information that identifies you in the body of your comments, that information will be posted on <https://www.regulations.gov>.
- If you want to submit a comment with confidential information that you do not wish to be made available to the public, submit the comment as a written/paper submission and in the manner detailed (see "Written/Paper Submissions" and "Instructions").

Written/Paper Submissions

Submit written/paper submissions as follows:

- Mail/Hand delivery/Courier (for written/paper submissions): Dockets Management Staff (HFA-305), Food and Drug Administration, 5630 Fishers Lane, Rm. 1061, Rockville, MD 20852.
- For written/paper comments submitted to the Dockets Management Staff, FDA will post your comment, as well as any attachments, except for information submitted, marked and identified, as confidential, if submitted as detailed in "Instructions."

Instructions: All submissions received must include the Docket No. FDA-2019-N-5646 for "FDA Rare Disease Day 2020: Supporting the Future of Rare Disease Product Development." Received comments, those filed in a timely manner (see ADDRESSES), will be placed in the docket and, except for those submitted as "Confidential Submissions," publicly viewable at <https://www.regulations.gov> or at the Dockets Management Staff between 9 a.m. and 4 p.m., Monday through Friday.

- Confidential Submissions--To submit a comment with confidential information that you do not wish to be made publicly available, submit your comments only as a written/paper submission. You should submit two copies total. One copy will include the information you claim to be confidential with a heading or cover note that states "THIS DOCUMENT CONTAINS CONFIDENTIAL INFORMATION." The Agency will review this copy, including the claimed confidential information, in its consideration of comments. The second copy, which will have the claimed confidential information redacted/blacked out, will be available for public viewing and posted on <https://www.regulations.gov>. Submit both copies to the Dockets Management Staff. If you do not wish your name and contact

information to be made publicly available, you can provide this information on the cover sheet and not in the body of your comments and you must identify this information as "confidential." Any information marked as "confidential" will not be disclosed except in accordance with 21 CFR 10.20 and other applicable disclosure law. For more information about FDA's posting of comments to public dockets, see 80 FR 56469, September 18, 2015, or access the information at <https://www.govinfo.gov/content/pkg/FR-2015-09-18/pdf/2015-23389.pdf>.

Docket: For access to the docket to read background documents or the electronic and written/paper comments received, go to <https://www.regulations.gov> and insert the docket number, found in brackets in the heading of this document, into the "Search" box and follow the prompts and/or go to the Dockets Management Staff, 5630 Fishers Lane, Rm. 1061, Rockville, MD 20852.

FOR FURTHER INFORMATION, CONTACT: Eleanor Dixon-Terry, Office of Orphan Products Development, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 32, Rm. 5163, Silver Spring, MD 20993, 301-796-7634, OOPDOrphanEvents@fda.hhs.gov.

SUPPLEMENTARY INFORMATION:

I. Background

Rare diseases, often referred to as orphan diseases, are diseases that affect less than 200,000 persons in the United States. While these diseases are individually rare, collectively they are not rare. There are more than 7,000 rare diseases affecting an estimated 30 million people in the United States. Many of these rare diseases are serious or life-threatening and many affect children.

The combination of government incentives and scientific advances has fueled extraordinary development in orphan drugs. Since the Orphan Drug Act was first passed in 1983, drugs and biologics for over 800 rare disease indications have been developed and approved for marketing. In addition to drugs and biologics, there has been progress in the development of medical devices for rare diseases. Since the implementation of the Humanitarian Use Device program in 1996, FDA has approved 77 medical devices for an orphan indication under the Agency's Humanitarian Device Exemption program. Unfortunately, most rare diseases still do not have approved treatments.

Developing a treatment for a rare disease can present unique challenges. Potential challenges include the small number of individuals affected, lack of understanding of the natural history of the disease, phenotypic heterogeneity, and lack of validated endpoints for use in clinical trials. Overcoming these challenges requires the collaboration between many stakeholders, including scientists, product developers, regulators, policy makers, and patients. In addition, as scientific understanding and technological development advances, it is essential for these stakeholders to stay abreast of the new challenges and opportunities in rare disease product development. Some challenges that need to be addressed include consideration of manufacturing needs to support the development of novel products, such as gene therapies, and considerations related to products developed for diseases or conditions affecting one or a few individuals. FDA is committed to working with stakeholders to advance treatment options for patients with rare diseases.

On April 29, 2019, FDA held a public meeting focusing on the perspectives of those affected by rare diseases. The FDA Rare Disease Day 2020 meeting will build on the previous meeting and include perspectives from additional stakeholders in rare disease product

development, such as academic investigators and pharmaceutical companies. While the differences between rare diseases are critically important, this meeting will look to find commonalities that may help the Agency and medical product developers further understand and advance the development of treatments for rare diseases. The specific goal of this upcoming meeting is to identify challenges and solutions in rare disease product development to optimize rare disease medical product development. Potential ways to accomplish this goal may include identifying common clinical trial designs and analytical plans for natural history or registry studies that would be applicable to many rare diseases.

This meeting will include participation of FDA, the patient community, patient advocacy groups, academic investigators, medical product developers, and other interested stakeholders.

II. Topics for Discussion at the Public Meeting

This public meeting will consist of presentations and interactive panel discussions. The presentations will provide information to outline different perspectives in rare disease product development. The panel discussions will be moderated and allow additional panelists to provide individual perspectives. There will be an opportunity for discussion between the panelists and the audience.

The meeting will focus on several related topics. First, FDA would like to hear from rare disease stakeholders on strategies to optimize registry and natural history data collection to support rare disease product development. Second, FDA would like to hear from rare disease stakeholders on new opportunities and challenges in rare disease product development in the setting of recent scientific advancements that may enable the development of medical products for diseases or conditions affecting one or a few individuals. FDA staff will also offer their perspective on these topics. We invite the public to register and participate in the public

meeting. A detailed agenda will be posted on the following website in advance of the meeting:

<https://www.fda.gov/news-events/fda-meetings-conferences-and-workshops/fda-rare-disease-day-2020-supporting-future-rare-disease-product-development-02242020-02242020>.

III. Participating in the Public Meeting

Registration: To register for the public meeting, please visit the following website by February 17, 2020: <https://www.eventbrite.com/e/supporting-the-future-of-rare-disease-product-development-public-meeting-registration-77190744595>. Please provide complete contact information for each attendee, including name, title, affiliation, address, email, and telephone.

Registration is free and based on space availability, with priority given to early registrants. Persons interested in attending this public meeting must register by February 17, 2020, 5 p.m. Eastern Time. Early registration is recommended because seating is limited; therefore, FDA may limit the number of participants from each organization. Registrants will receive confirmation when their registration has been received. If time and space permit, onsite registration on the day of the public meeting will be provided beginning an hour prior to the start of the meeting.

If you need special accommodations due to a disability, please contact Eleanor Dixon-Terry, at 301-796-7634, or OOPDOrphanEvents@fda.hhs.gov no later than February 17, 2020.

An agenda for the meeting and any other background materials will be made available 5 days before the meeting at <https://www.fda.gov/news-events/fda-meetings-conferences-and-workshops/fda-rare-disease-day-2020-supporting-future-rare-disease-product-development-02242020-02242020>.

Requests for Open Public Comment Period Speakers: FDA will hold an open public comment period to give the public an opportunity to comment on the meeting topics.

Registration for open public comment will occur in the meeting registration and at the registration desk on the day of the meeting on a first-come, first-served basis. The open public comment period is for in-person attendees only.

Open public comment period speakers will be notified of their selection approximately 7 days before the public meeting. We will try to accommodate all who wish to speak, either through the open public comment period or audience participation during the meeting; however, the duration of comments may be limited by time constraints.

Streaming Webcast of the Public Meeting: For those unable to attend in person, FDA will provide a live webcast of the meeting. To register for the streaming webcast of the public meeting, please visit the following website by February 23, 2020: <https://www.fda.gov/news-events/fda-meetings-conferences-and-workshops/fda-rare-disease-day-2020-supporting-future-rare-disease-product-development-02242020-02242020>.

If you have never attended a FDA York Media event before, test your connection at <http://www.yorkmedia.com/webcast/systemrequirements/>. FDA has verified the website addresses in this document, as of the date this document publishes in the *Federal Register*, but websites are subject to change over time.

Transcripts: Please be advised that as soon as a transcript of the public meeting is available, it will be accessible at <https://www.regulations.gov>. It may be viewed at the Dockets Management Staff (see ADDRESSES). A link to the transcript will also be available on the internet at <https://www.fda.gov/news-events/fda-meetings-conferences-and-workshops/fda-rare-disease-day-2020-supporting-future-rare-disease-product-development-02242020-02242020>.

Dated: January 13, 2020.

Lowell J. Schiller,

Principal Associate Commissioner for Policy.

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